STIC Biotechnology Systems Branch

RAW SEQUENCE LISTING ERROR REPORT

The Biotechnology Systems Branch of the Scientific and Technical Information Center (STIC) detected errors when processing the following computer readable form:

Application Serial Number: 10/552,287
Source: PCT/10
Date Processed by STIC: 10/18/05

THE ATTACHED PRINTOUT EXPLAINS DETECTED ERRORS.
PLEASE FORWARD THIS INFORMATION TO THE APPLICANT BY EITHER:
1) **INCLUDING A COPY OF THIS PRINTOUT IN YOUR NEXT COMMUNICATION TO THE APPLICANT, WITH A NOTICE TO COMPLY or,**
2) **TELEPHONING APPLICANT AND FAXING A COPY OF THIS PRINTOUT, WITH A NOTICE TO COMPLY**

FOR CRF SUBMISSION AND PATENTIN SOFTWARE QUESTIONS, PLEASE CONTACT MARK SPENCER, TELEPHONE: 571-272-2510; FAX: 571-273-0221

TO **REDUCE** ERRORED SEQUENCE LISTINGS, **PLEASE** USE THE **CHECKER VERSION 4.2.2 PROGRAM**, ACCESSIBLE THROUGH THE U.S. PATENT AND TRADEMARK OFFICE WEBSITE. SEE BELOW FOR ADDRESS:

http://www.uspto.gov/web/offices/pac/checker/chkrnote.htm

Applicants submitting genetic sequence information electronically on diskette or CD-Rom should be aware that there is a possibility that the disk/CD-Rom may have been affected by treatment given to all incoming mail.
Please consider using alternate methods of submission for the disk/CD-Rom or replacement disk/CD-Rom.
Any reply including a sequence listing in electronic form should NOT be sent to the 20231 zip code address for the United States Patent and Trademark Office, and instead should be sent via the following to the indicated addresses:

1. EFS-Bio (<**http://www.uspto.gov/ebc/efs/downloads/documents.htm**> , **EFS Submission User Manual** - ePAVE)
2. **U.S. Postal Service:** Commissioner for Patents, P.O. Box 1450, Alexandria, VA 22313-1450
3. **Hand Carry, Federal Express, United Parcel Service, or other delivery service (EFFECTIVE 01/14/05):** U.S. Patent and Trademark Office, Mail Stop Sequence, Customer Window, Randolph Building, 401 Dulany Street, Alexandria, VA 22314

Revised 01/24/05

Raw Sequence Listing Error Summary

ERROR DETECTED **SUGGESTED CORRECTION** SERIAL NUMBER: 10/552,287

ATTN: NEW RULES CASES: PLEASE DISREGARD ENGLISH "ALPHA" HEADERS, WHICH WERE INSERTED BY PTO SOFTWARE

1	Wrapped Nucleics Wrapped Aminos	The number/text at the end of each line "wrapped" down to the next line. This may occur if your file was retrieved in a word processor **after** creating it. Please adjust your right margin to .3; this will prevent "wrapping."
2	Invalid Line Length	The rules require that a line **not exceed** 72 characters in length. This includes white spaces.
3	Misaligned Amino Numbering	The numbering under each 5th amino acid is misaligned. Do **not** use tab codes between numbers; use **space characters**, instead.
4	Non-ASCII	The submitted file was **not** saved in ASCII(DOS) text, as **required** by the Sequence Rules. **Please ensure your subsequent submission is saved in ASCII text.**
5	Variable Length	Sequence(s)____contain n's or Xaa's representing more than one residue. **Per Sequence Rules, each n or Xaa can only represent a single residue.** Please present the **maximum** number of each residue having variable length and indicate in the <220>-<223> section that some may be missing.
6	PatentIn 2.0 "bug"	A "bug" in PatentIn version 2.0 has caused the <220>-<223> section to be missing from amino acid sequences(s) _____. Normally, PatentIn would automatically generate this section from the previously coded nucleic acid sequence. Please manually copy the relevant <220>-<223> section to the subsequent amino acid sequence. **This applies to the mandatory <220>-<223> sections for Artificial or Unknown sequences.**
7	Skipped Sequences (OLD RULES)	Sequence(s) _____ missing. If intentional, please insert the following lines for **each** skipped sequence: (2) INFORMATION FOR SEQ ID NO:X: (insert SEQ ID NO where "X" is shown) (i) SEQUENCE CHARACTERISTICS: (Do not insert any subheadings under this heading) (xi) SEQUENCE DESCRIPTION:SEQ ID NO:X: (insert SEQ ID NO where "X" is shown) This sequence is intentionally skipped Please also adjust the "(ii) NUMBER OF SEQUENCES:" response to **include** the skipped sequences.
8	Skipped Sequences (NEW RULES)	Sequence(s) _____ missing. If **intentional**, please insert the following lines for **each** skipped sequence. <210> sequence id number <400> sequence id number 000
9	Use of n's or Xaa's (NEW RULES)	Use of n's and/or Xaa's have been detected in the Sequence Listing. Per 1.823 of Sequence Rules, use of <220>-<223> is MANDATORY if n's or Xaa's are present. In <220> to <223> section, please explain location of **n** or **Xaa**, and which residue **n** or **Xaa** represents.
10	Invalid <213> Response	Per 1.823 of Sequence Rules, the only **valid** <213> responses are: Unknown, Artificial Sequence, or scientific name (Genus/species). <220>-<223> section is **required** when <213> response is Unknown or is Artificial Sequence
11	Use of <220>	Sequence(s) _____ missing the <220> "Feature" and associated numeric identifiers and responses. Use of <220> to <223> is MANDATORY if <213> "Organism" response is "Artificial Sequence" or "Unknown." Please explain source of genetic material in <220> to <223> section. (See "Federal Register," 06/01/1998, Vol. 63, No. 104, pp. 29631-32) (Sec. 1.823 of Sequence Rules)
12	PatentIn 2.0 "bug"	Please do not use "Copy to Disk" function of PatentIn version 2.0. This causes a corrupted file, resulting in missing mandatory numeric identifiers and responses (as indicated on raw sequence listing). Instead, please use "File Manager" or any other manual means to copy file to floppy disk.
13	Misuse of n/Xaa	"n" can **only** represent a single nucleotide; "Xaa" can **only** represent a single amino acid

PCT

RAW SEQUENCE LISTING DATE: 10/18/2005
PATENT APPLICATION: **US/10/552,287** TIME: 10:13:33

Input Set : **A:\PTO.RJ.txt**
Output Set: **N:\CRF4\10182005\J552287.raw**

3 <110> APPLICANT: Futerman, Anthony
4 Sussman, Joel
5 Silman, Israel
6 Harel, Michal
7 Dvir, Hay
8 Toker, Lilly
9 Swetlana Adamsky

see item 4 on Error Summary Sheet

11 <120> TITLE OF INVENTION: GAUCHER DISEASE DRUGS AND METHODS OF IDENTIFYING SAME
13 <130> FILE REFERENCE: 30227
C--> 15 <140> **CURRENT APPLICATION NUMBER: US/10/552,287**
C--> 15 <141> **CURRENT FILING DATE: 2005-10-04**
15 <160> NUMBER OF SEQ ID NOS: 16
17 <170> SOFTWARE: PatentIn version 3.2

ERRORED SEQUENCES

Does Not Comply
Corrected Diskette Needed

p.2

2007 <210> SEQ ID NO: 16
2008 <211> LENGTH: 497
2009 <212> TYPE: PRT
2010 <213> ORGANISM: Pan troglodytes
2012 <400> SEQUENCE: 16

```
2014 Ala Arg Pro Cys Ile Pro Lys Ser Phe Gly Tyr Ser Ser Val Val Cys
2015 1               5                  10                  15
2018 Val Cys Asn Ala Thr Tyr Cys Asp Ser Phe Asp Pro Pro Thr Phe Pro
2019             20                  25                  30
2022 Ala Leu Gly Thr Phe Ser Arg Tyr Glu Ser Thr Arg Ser Gly Arg Arg
2023         35                  40                  45
2026 Met Glu Leu Ser Met Gly Thr Ile Gln Ala Asn His Thr Gly Thr Gly
2027     50                  55                  60
2030 Leu Leu Leu Thr Leu Gln Pro Glu Gln Lys Phe Gln Lys Val Lys Gly
2031 65                  70                  75                  80
2034 Phe Gly Gly Ala Met Thr Asp Ala Ala Ala Leu Asn Ile Leu Ala Leu
2035                 85                  90                  95
2038 Ser Pro Pro Ala Gln Asn Leu Leu Leu Lys Ser Tyr Phe Ser Glu Glu
2039             100                 105                 110
2042 Gly Ile Gly Tyr Asn Ile Ile Arg Val Pro Met Ala Ser Cys Asp Phe
2043         115                 120                 125
2046 Ser Ile Arg Thr Tyr Thr Tyr Ala Asp Thr Pro Asp Asp Phe Gln Leu
2047     130                 135                 140
2050 His Asn Phe Ser Leu Pro Glu Glu Asp Thr Lys Leu Lys Ile Pro Leu
2051 145                 150                 155                 160
2054 Ile His Arg Ala Leu Gln Leu Ala Gln Arg Pro Val Ser Leu Leu Ala
```

RAW SEQUENCE LISTING DATE: 10/18/2005
PATENT APPLICATION: US/10/552,287 TIME: 10:13:33

Input Set : A:\PTO.RJ.txt
Output Set: N:\CRF4\10182005\J552287.raw

```
2055                 165                 170                 175
2058 Ser Pro Trp Thr Ser Pro Thr Trp Leu Lys Thr Asn Gly Ala Val Asn
2059             180                 185                 190
2062 Gly Lys Gly Ser Leu Lys Gly Gln Pro Gly Asp Ile Tyr His Gln Thr
2063         195                 200                 205
2066 Trp Ala Arg Tyr Phe Val Lys Phe Leu Asp Ala Tyr Ala Glu His Lys
2067     210                 215                 220
2070 Leu Gln Phe Trp Ala Val Thr Ala Glu Asn Glu Pro Ser Ala Gly Leu
2071 225                 230                 235                 240
2074 Leu Ser Gly Tyr Pro Phe Gln Cys Leu Gly Phe Thr Pro Glu His Gln
2075                 245                 250                 255
2078 Arg Asp Phe Ile Ala Arg Asp Leu Gly Pro Thr Leu Ala Asn Ser Thr
2079             260                 265                 270
2082 His His Asn Val Arg Leu Leu Met Leu Asp Asp Gln Arg Leu Leu Leu
2083         275                 280                 285
2086 Pro His Trp Ala Lys Val Val Leu Thr Asp Pro Glu Ala Ala Lys Tyr
2087     290                 295                 300
2090 Val His Gly Ile Ala Val His Trp Tyr Leu Asp Phe Leu Ala Pro Ala
2091 305                 310                 315                 320
2094 Lys Ala Thr Leu Gly Glu Thr His Arg Leu Phe Pro Asn Thr Met Leu
2095                 325                 330                 335
2098 Phe Ala Ser Glu Ala Cys Val Gly Ser Lys Phe Trp Glu Gln Ser Val
2099             340                 345                 350
2102 Arg Leu Gly Ser Trp Asp Arg Gly Met Gln Tyr Ser His Ser Ile Ile
2103         355                 360                 365
2106 Thr Asn Leu Leu Tyr His Val Val Gly Trp Thr Asp Trp Asn Leu Ala
2107     370                 375                 380
2110 Leu Asn Pro Glu Gly Gly Pro Asn Trp Val Arg Asn Phe Val Asp Ser
2111 385                 390                 395                 400
2114 Pro Ile Ile Val Asp Ile Thr Lys Asp Thr Phe Tyr Lys Gln Pro Met
2115                 405                 410                 415
2118 Phe Tyr His Leu Gly His Phe Ser Lys Phe Ile Pro Glu Gly Ser Gln
2119             420                 425                 430
2122 Arg Val Gly Leu Val Ala Ser Gln Lys Asn Asp Leu Asp Ala Val Ala
2123         435                 440                 445
2126 Leu Met His Pro Asp Gly Ser Ala Val Val Val Val Leu Asn Arg Ser
2127     450                 455                 460
2130 Ser Lys Asp Val Pro Leu Thr Ile Lys Asp Pro Ala Val Gly Phe Leu
2131 465                 470                 475                 480
2134 Glu Thr Ile Ser Pro Gly Tyr Ser Ile His Thr Tyr Leu Trp Arg Arg
2135                 485                 490                 495
2138 Gln
E--> 2147 (1) delete
```

see pp 3, 5-6

<210> 7
<211> 497
<212> PRT
<213> Homo sapiens

<220>
<221> misc_feature
<222> (496)..(496)
<223> Arg to His mutant

(why is "Arg" listed in location 496 instead of "His"? see p5)

<400> 7

Ala Arg Pro Cys Ile Pro Lys Ser Phe Gly Tyr Ser Ser Val Val Cys
1 5 10 15

Val Cys Asn Ala Thr Tyr Cys Asp Ser Phe Asp Pro Pro Thr Phe Pro
20 25 30

Ala Leu Gly Thr Phe Ser Arg Tyr Glu Ser Thr Arg Ser Gly Arg Arg
35 40 45

Met Glu Leu Ser Met Gly Pro Ile Gln Ala Asn His Thr Gly Thr Gly
50 55 60

Leu Leu Leu Thr Leu Gln Pro Glu Gln Lys Phe Gln Lys Val Lys Gly
65 70 75 80

Phe Gly Gly Ala Met Thr Asp Ala Ala Ala Leu Asn Ile Leu Ala Leu
85 90 95

Ser Pro Pro Ala Gln Asn Leu Leu Leu Lys Ser Tyr Phe Ser Glu Glu
100 105 110

Gly Ile Gly Tyr Asn Ile Ile Arg Val Pro Met Ala Ser Cys Asp Phe
115 120 125

Ser Ile Arg Thr Tyr Thr Tyr Ala Asp Thr Pro Asp Asp Phe Gln Leu
130 135 140

His Asn Phe Ser Leu Pro Glu Glu Asp Thr Lys Leu Lys Ile Pro Leu
145 150 155 160

Ile His Arg Ala Leu Gln Leu Ala Gln Arg Pro Val Ser Leu Leu Ala
165 170 175

```
Ser Pro Trp Thr Ser Pro Thr Trp Leu Lys Thr Asn Gly Ala Val Asn
            180                 185                 190

Gly Lys Gly Ser Leu Lys Gly Gln Pro Gly Asp Ile Tyr His Gln Thr
        195                 200                 205

Trp Ala Arg Tyr Phe Val Lys Phe Leu Asp Ala Tyr Ala Glu His Lys
    210                 215                 220

Leu Gln Phe Trp Ala Val Thr Ala Glu Asn Glu Pro Ser Ala Gly Leu
225                 230                 235                 240

Leu Ser Gly Tyr Pro Phe Gln Cys Leu Gly Phe Thr Pro Glu His Gln
                245                 250                 255

Arg Asp Phe Ile Ala Arg Asp Leu Gly Pro Thr Leu Ala Asn Ser Thr
            260                 265                 270

His His Asn Val Arg Leu Leu Met Leu Asp Asp Gln Arg Leu Leu Leu
        275                 280                 285

Pro His Trp Ala Lys Val Val Leu Thr Asp Pro Glu Ala Ala Lys Tyr
    290                 295                 300

Val His Gly Ile Ala Val His Trp Tyr Leu Asp Phe Leu Ala Pro Ala
305                 310                 315                 320

Lys Ala Thr Leu Gly Glu Thr His Arg Leu Phe Pro Asn Thr Met Leu
                325                 330                 335

Phe Ala Ser Glu Ala Cys Val Gly Ser Lys Phe Trp Glu Gln Ser Val
            340                 345                 350

Arg Leu Gly Ser Trp Asp Arg Gly Met Gln Tyr Ser His Ser Ile Ile
        355                 360                 365

Thr Asn Leu Leu Tyr His Val Val Gly Trp Thr Asp Trp Asn Leu Ala
    370                 375                 380

Leu Asn Pro Glu Gly Gly Pro Asn Trp Val Arg Asn Phe Val Asp Ser
385                 390                 395                 400

Pro Ile Ile Val Asp Ile Thr Lys Asp Thr Phe Tyr Lys Gln Pro Met
```

405 410 415

Phe Tyr His Leu Gly His Phe Ser Lys Phe Ile Pro Glu Gly Ser Gln
420 425 430

Arg Val Gly Leu Val Ala Ser Gln Lys Asn Asp Leu Asp Ala Val Ala
435 440 445

Leu Met Asn Pro Asp Gly Ser Ala Val Val Val Val Leu Asn Arg Ser
450 455 460

Ser Lys Asp Val Pro Leu Thr Ile Lys Asp Pro Ala Val Gly Phe Leu
465 470 475 480

Glu Thr Ile Ser Pro Gly Tyr Ser Ile His Thr Tyr Leu Trp His Arg
485 490 495

same error in Sequence 14

<210> 9
<211> 497
<212> PRT
<213> Homo sapiens

<220>
<221> misc_feature
<222> (370)..(370)
<223> Asn to Ser mutant

VERIFICATION SUMMARY DATE: 10/18/2005
PATENT APPLICATION: **US/10/552,287** TIME: 10:13:34

Input Set : **A:\PTO.RJ.txt**
Output Set: **N:\CRF4\10182005\J552287.raw**

L:15 M:270 C: Current Application Number differs, Replaced Current Application No
L:15 M:271 C: Current Filing Date differs, Replaced Current Filing Date
L:2002 M:341 W: (46) "n" or "Xaa" used, for SEQ ID#:15 after pos.:0
L:2147 M:332 E: (32) Invalid/Missing Amino Acid Numbering, SEQ ID:16